What Are Clinical Trials All About?

A Booklet for Patients with Cancer

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What Are Clinical Trials All About?

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What Are Clinical Trials All About?

Foreword

Research studies conducted with patients are called clinical trials. As a cancer patient, you may take part in a clinical trial. This booklet is written for you, your family and friends, to explain what clinical trials are and to help you make a decision about entering a trial.

The time when cancer is diagnosed or when treatment decisions are being made is very difficult. It is often hard to understand or remember complex medical explanations. The information in this booklet is meant to supplement what your doctors tell you. It provides answers to questions asked most often about clinical trials. (You may not want to read the whole book at one time. It is broken down into questions and answers that you can read now or later.)

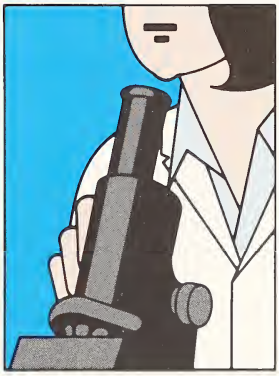
There is a page labeled "Notes" near the back. You may wish to write down questions to ask your doctor or nurse. Also, there is a glossary of words that relate to clinical trials and cancer care. This is a quick way to look up terms that you may hear or read. More information on many cancer-related topics is available at no cost in other publications from the National Cancer Institute (see back cover).

We hope this booklet will help to explain how clinical studies are designed and carried out. Of course, there are good treatments and good care for cancer patients whether they take part in clinical trials or receive standard treatments. You may decide not to take part in a trial, and you can still receive good medical care. The decision to enter a clinical trial or not is always up to you.

What Is a Clinical Trial?

In cancer research, a clinical trial is a study conducted with cancer patients, usually to evaluate a new treatment. Each study is designed to answer scientific questions and to find new and better ways to help cancer patients.

The search for good cancer treatments begins with basic research in laboratory and animal studies. The best results of that research are tried in patient studies, hopefully leading to findings that may help many people.



Before a new treatment is tried with patients, it is carefully studied in the laboratory. This research points out the new methods most likely to succeed, and, as much as possible, shows how to use them safely and effectively. But this early research cannot predict exactly how a new treatment will work with patients.

With any new treatment there may be risks as well as possible benefits. There may also be some risks that are not yet known. Clinical trials help us find out if a promising new treatment is safe and effective for patients. During a trial, more and more information is gained about a new treatment, its risks, and how well it may or may not work.

Standard treatments, the ones now being used, are often the base for building new, hopefully better treatments. Many new treatments are designed on the basis of what has worked in the past, in efforts to improve on this.

Only patients who wish to, take part in a clinical trial. You may be interested in or asked to enter a trial. Learn as much as you can about the trial, before you make up your mind.

Why Are Clinical Trials Important?

Advances in medicine and science are the results of new ideas and approaches developed through research. New cancer treatments must prove to be safe and effective in scientific studies with a certain number of patients before they can be made widely available.

Through clinical trials, researchers learn which approaches are more effective than others. This is the best way to test a new treatment. A number of standard treatments were first shown to be effective in clinical trials. These trials helped to find new and better treatments.

Why Would a Patient Be Interested in a Clinical Trial?

Patients take part in clinical trials for many reasons. Usually, they hope for benefits for themselves. They may hope for a cure of disease, a longer time to live, a way to feel better. Often they want to contribute to a research effort that may help others.

Based on what researchers learn from laboratory studies, and sometimes earlier clinical studies and standard treatments as well, they design a trial to see if a new treatment will improve on current treatments. The hope is that it will. Often researchers use standard treatments as the building blocks to try to design better treatments.

Although there is always a chance that a new treatment will be disappointing, the researchers involved in a study have reason to believe that it will be as good as, or better than, current treatments.

The patients in a clinical trial are among the first to receive new research treatments before they are widely available. How a treatment will work for a patient in a trial can't be known ahead of time. Even standard treatments, although effective in many patients, do not carry sure benefits for everyone. But patients should choose if they want to take part in a study or not, only after they understand both the possible risks and benefits.

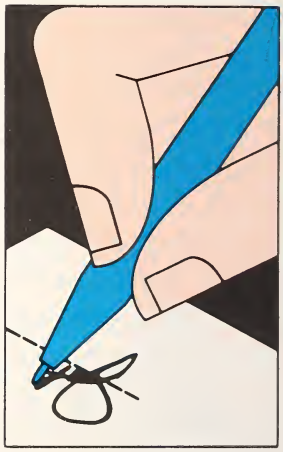
The patients who take part in clinical trial procedures that do prove to be better treatments have the first chance to benefit from them. All patients in clinical trials are carefully monitored during a trial and followed up afterwards. They become part of a network of clinical trials carried out around the country. In this network, doctors and researchers pool their ideas and experience to design and monitor clinical studies. They share their knowledge from many specialties about cancer treatment and care. Patients in these studies receive the benefit of their expertise. At cancer centers, patients receive care from a special research team. Through new programs, community hospitals and doctors are also coming more and more into the research network.

Are There Risks or Side Effects in Clinical Trials?

Yes. The treatments used in clinical trials can cause side effects and other health risks depending on the type of treatment and the patient's condition. Side effects vary from patient to patient.

Because clinical trials are research into new areas of treatment, the risks involved are not always known ahead of time, though efforts have been made to find out what they might be. For this reason, trials can carry unknown dangers and side effects as well as hoped-for benefits.

Patients need to know what is involved in a study—what side effects may be expected—and, as much as possible, what unknowns or uncertainties they may be facing.



Your doctor or nurse will tell you about the treatments being tested and will give you a form to read that discusses the risks and hoped-for benefits. If you agree to take part, you will be asked to sign a form, called the informed consent form. Before you sign, be sure you understand what risks you face. Ask the doctor or nurse to explain any parts of the form or the trial that are not clear. If you do not want to be in the trial, you may refuse. Even if you sign the form, you are free to leave the trial at any time and can receive other available medical care.

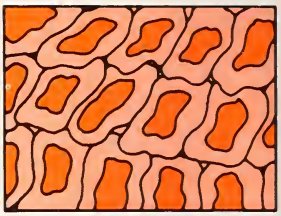
In clinical trials, most side effects are temporary and will gradually go away once

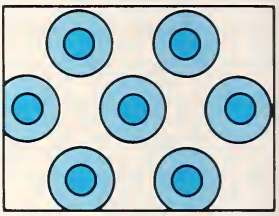
treatment is stopped. For example, some anticancer drugs cause hair loss and nausea and some do not. They can also affect the bone marrow which produces blood cells. During treatment, the number of blood cells, called blood counts, may fall too low. Since this could lead to possible infection or other problems, patients have their blood counts checked often. Luckily, bone marrow has a great ability to replace blood cells, so that blood counts can usually return to normal.

Some side effects in clinical trials can be permanent and serious, even life-threatening. Also, certain side effects may not appear until later, after the treatment itself is over. (These "late" effects may include damage to a major organ like the heart, lungs, or kidneys; sterility; or a second cancer.) Many cancer patients are now living longer, largely because of better treatments. Researchers are concerned and trying to prevent late complications of treatments.

As a patient, it can be hard to decide about your treatment. There are a number of things to consider. Cancer is a life-threatening disease which causes symptoms of its own that are not related to treatment. In each case, the unavoidable risks of the cancer itself, and your condition, should be weighed against the potential risks and benefits of a new research treatment. Standard treatments, as well as treatments in clinical trials, can also cause side effects and risks.

Why Does Cancer Treatment Have Side Effects?





Any medical treatment can carry the potential for side effects in some patients. Cancer treatment is particularly powerful, because it is designed to destroy constantly dividing cancer cells. It can also affect healthy dividing cells and this can cause side effects. The challenge to researchers has been to develop treatments that destroy cancer cells but do not harm healthy cells.

What Is Being Done To Lessen Side Effects of Treatment?

Cancer researchers are trying to make cancer treatment more effective and lessen its side effects for the cancer patient. Results of such efforts include:

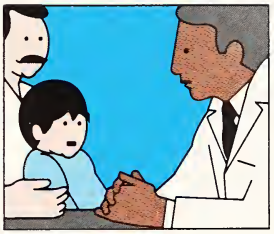
- new anticancer drugs with less side effects;
- better antinausea medicine;
- some shorter periods of time on anticancer drugs;
- special ways to protect normal tissues during radiation therapy;
- new methods of surgery that are less extensive and less damaging to the body; and
- psychological support programs and information on ways to cope during difficult times. How patients feel during and after treatment is important.

If You Are Thinking of Entering a Clinical Trial...

Are You Eligible for a Clinical Trial?

Every clinical trial is designed to answer a set of research questions. If you fit the guidelines for a trial, you may be eligible to take part. Each study enrolls patients with certain types and stages of cancer and certain health status. A study that involves two or more treatments can yield reliable answers only if all the patient cases are the same so they can be compared with each other.

Before you and your physician make a decision about your treatment (whether it is in a clinical trial or not), your type of cancer will be diagnosed and "staged." Staging tells how far the disease has spread. Deciding on treatment depends on many things, including the stage of the disease and your general health. You would most likely be referred to a trial by your own doctor or by a doctor who knows your case. Some patients find out about trials from other sources. In any case, you must have a reasonable understanding of your role in a research study and be freely willing to take part in it. Ask what you can expect if you take part in a trial.



What Trials Are Available for Your Type of Cancer?

There are many ways to find out what your treatment choices are. Talk with your doctors and get the opinion of cancer specialists

(oncologists). You should not be afraid to ask for a second opinion. A helpful treatment information system called PDQ is supported by the National Cancer Institute. PDQ can give your doctor the latest information on clinical trials being offered around the country for each type and stage of cancer.

This ready reference is kept up to date. Your doctor can check it from a medical library or personal computer.

The Cancer Information Service (CIS) is another source of information. This program, also sponsored by the National Cancer Institute, answers cancer-related questions from the public, cancer patients and their families, and health professionals. If you have questions, call the toll-free number: 1-800-4-CANCER and you will be connected to the CIS office serving your area. Spanish-speaking CIS staff are also available at this number.

What Is Best for You?

This is a big question. Finding answers and making decisions are often hard for a cancer patient. The diagnosis of cancer and deciding what to do about it can be overwhelming, and you may be confused and upset. It is important to discuss your options with medical experts—including your own doctor—and with those close to you. Your personal doctor, who may be your family doctor, and cancer specialists can counsel you about your choices for standard treatment or clinical trials.

Talk to them and ask questions about the problems you are facing. If you understand what is going on, you can help your doctor work with you more effectively. You may want to take a friend or relative along with you when you talk to your doctor about your case.

Take time to ask your questions and to discuss what you want to know. It may help you and your doctor if you plan what to ask and write questions down ahead of time. No question is foolish. Learn what is available to you. Find out your choices and the risks and benefits of each.

Each patient is different. You are an individual with individual needs, and your health is important. If you are a parent of a child with cancer, of course you have great concerns about making the best decision for your child's care.

As you decide about treatment, if it is in a clinical trial or not, remember that you are not alone. There are many people to help you—doctors, nurses, social workers, clergy, your family, friends, and other patients. Although is it YOUR decision, they can help you think about it and decide what is best for you.

What Are Important Questions To Ask About a Clinical Trial?

If you are thinking about taking part in a clinical trial, here are some important questions to ask:

- What is the purpose of the study?

- What does the study involve? What kinds of tests and treatments? (Find out what is done and how it is done.)
- What is likely to happen in my case with, or without, this new research treatment? (What may the cancer do and what may this treatment do?)
- What are other choices and their advantages and disadvantages? (Are there standard treatments for my case and how does the study compare with them?)
- How could the study affect my daily life?
- What side effects could I expect from the study? (There can also be side effects from standard treatments and from the disease itself.)
- How long will the study last? (Will it require an extra time commitment on my part?)
- Will I have to be hospitalized? If so, how often and for how long?
- Will I have any costs? Will any of the treatment be free?*
- If I am harmed as a result of the research, what treatment would I be entitled to?
- What type of long-term followup care is part of the study?

*Costs are a major concern of patients and families. Different arrangements and policies exist at different institutions and, of course, insurance coverage varies. Patients should freely discuss what costs are involved in their cases ahead of time. If you need financial aid, contact the hospital social services office, the Cancer Information Service, or the local American Cancer Society Chapter. They may be able to direct you to a source of help.

What Is Informed Consent?

Informed consent, a key part of a good trial, is required in studies that are federally regulated or funded as well as by many state laws. Informed consent means that as a patient, you are given information so you can understand what is involved in a trial, including its potential benefits and risks, and then decide freely to take part in it or not. The nature of the treatment is explained by the doctors and nurses in the trial. You are given an informed consent form to read and consider carefully. Ask any questions you may have. Then, if you agree to take part, you can sign the form. Of course, you can also refuse.

The informed consent process (also discussed on page 4) is an ongoing process. If you enter a trial, you will continue to receive any new information about your treatment that may affect your willingness to stay in the trial. Signing a consent form does not bind you to the study. You can still choose to leave the study at any time.

What Is It Like To Be a Patient in a Clinical Trial?

Whether cancer patients are in a research study or not, they face a new world of medical terms and procedures. For some people, myths and fears of "experimentation" or of being a "guinea pig" come with the idea of clinical trials. And, surely, there are fears of the unknown.

Understanding what is involved can ease some of your anxieties. Patients in a clinical trial, for example, receive their care in the same places that standard treatments are given—at cancer centers, hospitals, clinics, or doctors' offices.

Because a growing number of cancer specialists are now in private practice in the community, most cancer care can be given in an area near your home. Doctors, nurses, social workers and other health professionals from many different specialties may help care for you. They are working together for your good. There is consideration for your privacy and well-being.

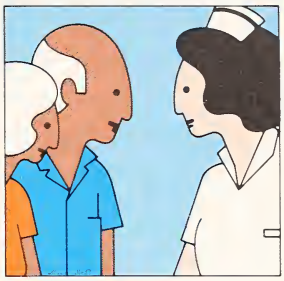
If you join a research study, you will be watched closely and data on your case will be carefully recorded. You may receive more examinations and tests than are usually given. (These are to follow your progress as well as to collect study data.) Of course, tests can carry certain risks and benefits or discomforts of their own. Although they can be inconvenient, these tests can assure an extra ounce of observation along the way.

During the course of a study, if it is clear that a treatment is not in your best interest, you will be removed from the study and you can discuss other options with your physician.

Can You Leave a Trial at Any Time?

Yes. Just as you can refuse to join a study, you may leave a study at any time. Your rights as an individual do not change because you are a patient in a clinical trial. You may choose to

take part or not, and you can always change your mind later, even after you enter a trial.



You may also refuse to take part in any aspect of the research. If you have questions at any time about any part of the study, be sure to ask your doctors. If you are not satisfied with the answers, you may consider leaving the study. If you decide to leave, it will not be held against you. You can freely discuss other possible treatments and care with your doctors and nurses.

What Protection Do You Have as a Patient in a Clinical Trial?

The ethical and legal codes that govern medical practice apply to clinical trials. In addition, most clinical research is federally regulated or federally funded (at least in part), with built-in safeguards to protect patients. These safeguards include regular review of the protocol (the study plans) and the progress of each study by researchers at other places.

For example, federally funded and federally regulated clinical trials must first be approved by an Institutional Review Board (IRB) located at the institution where the study is to take place. IRBs, designed to protect patients, are made up of scientists, doctors, clergy and other people from the local community. An IRB reviews a study to see that it is well designed with safeguards for patients and that the risks are reasonable in relation to the potential benefits.

Federally supported or regulated studies also go through reviews by a government agency,

such as the National Cancer Institute*, which sponsors and monitors many trials around the country.

Any well-run clinical trial, whether federally supported or not, is carefully reviewed for medical ethics, patient safety, and scientific merit by the research institution. Every study should provide for monitoring the data and the safety of patients on an ongoing basis.

As discussed earlier, informed consent is also an important process that helps to protect patients.

After patients join a clinical trial and it progresses, the doctors report the results of the trial to scientific meetings, to medical journals whose articles are approved by experts, and to various government agencies.

What Can Help You Learn If a Trial Is Sound and Well Run?

Things that make a sound, well-run trial that safeguards patients include the items discussed in the previous section of this booklet. Keeping these items in mind, here are some important questions for you to ask to find out if a study is well run:

- What is its purpose?

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*The National Cancer Institute (NCI) is the Federal Government's chief agency for cancer research. Located at the National Institutes of Health (NIH) in Bethesda, Maryland, the Institute funds cancer research across the country and conducts research at its own facilities. For information about NCI trials, call: 1-800-4-CANCER.

- Who has reviewed and approved the study?
- Who is sponsoring the study?
- How are the study data and patient safety being checked?
- Where will information from the study go? (In government-related research, for example, reports might go to the National Cancer Institute and/or the Food and Drug Administration.)

For your own protection, be sure to get satisfactory answers to these questions before you agree to take part.

What Kinds of Clinical Trials Are There?

There are many kinds of clinical trials. They range from studies of ways to prevent, detect and diagnose, control and treat cancer, to studies of the psychological impact of the disease and ways to improve the patient's comfort and quality of life (including pain control).

Most cancer clinical trials deal with new treatments. These treatments often involve surgery, radiation therapy (the use of X-rays, neutrons or other types of cell-destroying radiation), and chemotherapy (the use of anticancer drugs). Alone, or in combination, these types of treatments can cure many cancer patients and prolong the lives of many others. A fairly new area of cancer treatment is biological therapy—the use of biologicals (substances produced by the body's own cells) and biological response modifiers (substances that affect the body's natural defense systems against disease).

How Are Trials Divided Into Phases?

Clinical trials are carried out in phases, each designed to find out certain information. Patients may be eligible for studies in different phases depending on their general condition and the type and stage of their cancer. More patients take part in the later phases of studies than in the earlier ones.

In a Phase I study, a new research treatment is given to a small number of patients. The researchers must find the best way to give a new treatment and how much of it can be given safely. They watch carefully for any harmful side effects. The research treatment has been well tested in laboratory and animal studies but no one knows how patients will react. Phase I studies may involve significant risks for this reason. They are offered only to patients whose cancer has spread and who would not be helped by other known treatments. Phase I treatments may produce anticancer effects, and some patients have been helped by these treatments.

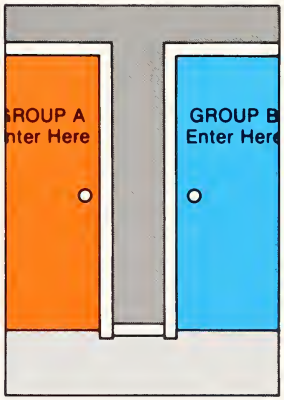
Phase II studies determine the effect of a research treatment on various types of cancer. Each new phase of a clinical trial depends on and builds on information from an earlier phase. If a treatment has shown activity against cancer in Phase II, it moves to Phase III. Here it is compared with standard treatment to see which is more effective. Often researchers use standard therapy as the base to design new, hopefully better treatments. Then in Phase III, the new

treatment is directly compared to the old one. In Phase IV studies, the new research treatment becomes part of standard treatment in patient care. For example, a new drug that has been found effective in a clinical trial may then be used together with other effective drugs, or with surgery and/or radiation therapy.

How Are Clinical Trials Conducted?

The doctors who conduct a clinical trial follow a carefully designed treatment plan called a "protocol." This spells out what will be done and why. Studies are planned to safeguard the medical and psychological health of patients as well as to answer research questions.

Some clinical trials test one research treatment in one group of patients. Other trials compare two or more treatments in separate groups of patients who are similar in certain ways, such as the extent of their disease. This way, the treatment groups are alike and the results from each can validly be compared.



One of the groups may receive standard (the most accepted) treatment so the new treatments can be directly compared to it. The group receiving the standard treatment is called the "control" group. For example, one group of patients (the control group) may receive the usual surgical treatment for a certain cancer, while another patient group with the same type of cancer may receive surgery plus radiation therapy to see if this improves disease control.

Sometimes, no standard treatment yet exists for certain cancer patients. In drug studies for such cases, one group of patients might receive a new drug and the control group, none. But no patient is placed in a control group without treatment if there is any known treatment that would benefit that patient. The control group is followed as often and carefully as the "treatment" group.

One of the ways to prevent the bias of a patient or doctor from influencing study results is "randomization." If a patient agrees to be randomized, this means he or she is selected by chance to be in one group or another. The researchers do not know which treatment is best. From what is known at the time, any one of the treatments chosen could be of equal benefit to the patient.

If the treatment in a trial is not helping the patient, the patient's doctor can decide to take him or her out of the study. Of course, the patient can decide to leave, as well, and still receive other available care. There are regular reviews of the results of a trial and the information is shared. This is important, because if a treatment is found to be too harmful or not effective, it is stopped. Also, when there is firm evidence that one method is better than the others in a study, the trial is stopped and all patients in the trial are given the benefit of the new information. Such information may help present and future patients.

Throughout a clinical study, a patient's personal doctor will be kept informed of the patient's progress. Patients are encouraged to maintain contact with their referring doctors.

The National Cancer Program and Clinical Trials

A nationwide effort to conquer cancer intensified with the National Cancer Act of 1971. As a result of the National Cancer Program, created by that legislation, more cancer patients are being cured today than ever before, and many others are living longer with improved quality of life.

The National Cancer Program brings together a network of researchers at many public and private institutions around the country. These include the National Cancer Institute, cancer centers, universities, community hospitals and private industry. Groups involving hundreds of researchers are working to discover and put to use new knowledge to benefit the cancer patients of today and tomorrow.

Knowledge gained from research studies with patients—clinical trials—has been essential to overall progress. Such studies have led to increased survival for childhood cancers, Hodgkin's disease, breast, uterine, testicular and bladder cancers, as well as others. These studies continue to play a key role in progress against cancer.

Today, major scientific discoveries in the laboratory are part of a revolution in biology. New tools to unravel the process of cancer are leading to exciting new approaches against cancer. Clinical trials continue to be the link between such basic research and patients. The goal is to translate the best of that research into findings that directly help people.

Resources

Information about cancer is available from many sources, including the ones listed below. You may wish to check for additional information at your local library or bookstore and from support groups in your community.

Cancer Information Service

The Cancer Information Service, a program of the National Cancer Institute, is a nationwide telephone service for cancer patients and their families and friends, the public, and health care professionals. The staff can answer questions (in English or Spanish) and can send free National Cancer Institute booklets about cancer. They also know about local resources and services. One toll-free number, 1-800-4-CANCER (1-800-422-6237), connects callers all over the country with the office that serves their area.

American Cancer Society

The American Cancer Society is a voluntary organization with a national office and local units all over the country. This organization supports research, conducts educational programs, and offers many services to patients and their families. The American Cancer Society also provides free booklets on cancer. To obtain booklets, or for information about services and activities in local areas, call the Society's toll-free

number, 1-800-ACS-2345, or the number listed under American Cancer Society in the white pages of the telephone book.

PDQ

The National Cancer Institute has developed PDQ, a computerized database designed to give doctors quick and easy access to:

- the latest treatment information for most types of cancer (also available by fax machine; the fax number is 301-402-5874);
- descriptions of clinical trials that are open for enrollment (including the names and addresses of the physicians and facilities conducting the studies); and
- the names of organizations and physicians involved in cancer care.

To access PDQ, physicians may use an office computer with a telephone hookup and a PDQ access code, or the services of a medical library with online searching capability. Cancer Information Service offices (1-800-4-CANCER) provide PDQ searches to callers and can tell physicians how to obtain regular access to the database. Patients may ask their physicians to use PDQ or may call the Cancer Information Service at 1-800-4-CANCER to request a search for themselves. PDQ is updated monthly with the latest cancer information on prevention, diagnosis, treatment, and rehabilitation.

Glossary

Adjuvant Chemotherapy (ad'ju-vant kee-mo-ther'a-pee)—One or more anticancer drugs used in combination with surgery or radiation therapy as part of the treatment of cancer. Adjuvant usually means "in addition to" initial treatment.

Antibody (an'ti-bod-ee)—A protein produced by a plasma cell in the lymphatic system or bone marrow. An antibody binds to the specific antigen that has stimulated the immune system. Once bound, the antigen can be destroyed by other cells of the immune system. See **Immune System**.

Antigen (an'ti-jen)—A substance, foreign to the body, that stimulates the production of antibodies by the immune system. Antigens include foreign proteins, bacteria, viruses, pollen and other materials.

Biological Therapy—Use of biologicals (substances produced by our own cells) or biological response modifiers (substances that affect the patient's defense systems) in the treatment of cancer.

Blood Count—Measurement of the number of red cells, white cells, and platelets in a sample of blood.

Bone Marrow (mair'oh)—The inner, spongy core of bone that produces blood cells.

Cancer (kan'ser)—A general term for more than 100 diseases characterized by abnormal and uncontrolled growth of cells. The resulting mass, or tumor, can invade and destroy surrounding normal tissues. Cancer

cells from the tumor can spread through the blood or lymph to start new cancers in other parts of the body.

CCOP (Community Clinical Oncology Program)—This new program links community physicians with NCI clinical research programs, so that more cancer patients can participate in clinical trials in their own communities.

Chemotherapy (kee-mo-ther'a-pee)—Treatment with anticancer drugs.

Clinical Trial—The systematic investigation of the effects of materials or methods, according to a formal study plan and generally in a human population with a particular disease or class of diseases. In cancer research, a clinical trial generally refers to the evaluation of treatment methods, such as surgery, drugs, or radiation techniques, although methods of prevention, detection, or diagnosis also may be the subject of such studies.

Combination Chemotherapy (kee-mo-ther'a pee)—Use of two or more anticancer drugs.

Combination Therapy (ther'a-pee)—The use of two or more modes of treatment—surgery, radiotherapy, chemotherapy, immunotherapy—in combination, alternately or together, to achieve optimum results against cancer.

Control Group—In clinical studies, this is a group of patients that receives *standard treatment*, a treatment or intervention currently being used and considered to be of

proved effectiveness on the basis of past studies. Results in patients receiving newly developed treatments may then be compared to the control group. In cases where no standard treatment yet exists for a particular condition, the control group would receive no treatment. No patient is placed in a control group without treatment if there is any beneficial treatment known for that patient.

Double-blind—Characteristic of a controlled experiment in which neither the patient nor the attending physician knows whether the patient is getting one or another drug or dose. In *single-blind* studies, patients do not know which of several treatments they are receiving, thus preventing personal bias from influencing their reactions and study results. In either case, the treatment can be quickly identified, if necessary, by a special code.

Hormone—Chemical product of the endocrine glands of the body, which, when secreted into body fluids, has a specific effect on other organs.

Immune System—A complex network of organs, cells, and specialized substances distributed throughout the body and defending it from foreign invaders that cause infection or disease.

Immunotherapy (im-mew-no-ther'a-pee)—A form of biological therapy. An experimental method of treating cancer, using substances which stimulate the body's immune defense system.

Informed Consent—The process in which a patient learns about and understands the purpose and aspects of a clinical trial and then agrees to participate. Of course, a patient may decline to participate. This process includes a document defining how much a patient must know about the potential benefits and risks of therapy before being able to agree to undergo it knowledgeably. (Informed consent is required by federally conducted, funded, or regulated studies as well as by many state laws.) If a patient signs an informed consent form and enters a trial, he or she is still free to leave the trial at any time, and can receive other available medical care.

Interferon (in-tur-feer'on)—A protein substance produced by white blood cells and other types of cells that have been exposed to certain viruses. In test animals, interferon has shown some activity against tumors. Studies of its usefulness in treating some types of human cancer are under way. One of a number of new agents available as biological therapy.

Investigational New Drug—A drug allowed by the Food and Drug Administration (FDA) to be used in clinical trials but not approved by the FDA for commercial marketing.

Investigator—An investigator is the experienced clinical researcher who prepares a protocol or treatment plan and implements it with patients.

Metastasis (me-tas'ta-sis)—The transfer of disease from one part of the body to another. In cancer, metastasis is the migration of cancer cells from the original tumor site through the blood and lymph vessels to produce cancers in other tissues. Metastasis also is the term used for a secondary cancer growing at a distant site.

Metastatic Cancer (met-a-stat'ik)—Cancer that has spread from its original site to one or more additional body sites.

Monoclonal Antibodies (mon-o-klone' al an'ti-bod-eez)—One of several new substances used in biological therapy. These antibodies, all exactly alike, are mass-produced and designed to home in on target cancer cells. Monoclonal antibodies are products of new scientific techniques and may prove useful in both cancer diagnosis and treatment.

Multimodality Therapy (mul'ti-mo-dal'i-tee ther'a-pee)—The combined use of more than one method of treatment, for example, surgery and chemotherapy.

Oncologist (on-kol'o-jist)—A physician who is a cancer specialist.

PDQ—A computerized database supported by NCI that is available to physicians nationwide. Geographically matrixed, it offers the latest information on standard treatments and ongoing clinical trials for each type and stage of cancer. The information is easily accessible for physicians via libraries and personal computers.

Placebo (pla-see'bo)—An inactive substance resembling a medication, given for psychological effect or as a control in evaluating a medicine believed to be active. It is usually a tablet, capsule, or injection that contains a harmless substance but appears to be the same as the medicine being tested. A placebo may be compared with a new drug when no one knows if any drug or treatment will be effective.

Protocol (pro'to-kol)—The outline or plan for use of an experimental procedure or experimental treatment.

Radiation Therapy—Treatment using X-rays, cobalt-60, radium, neutrons, or other types of cell-destroying radiation (also called radiotherapy).

Radiosensitizers (ray'dee-o-sen-si-ty'zers)—Drugs being studied to try to boost the effect of radiation therapy.

Randomized Clinical Trials (ran-duh'mized)—A study in which patients with similar traits, such as extent of disease, are chosen or selected by chance to be placed in separate groups that are comparing different treatments. Because irrelevant factors or preferences do not influence the distribution of patients, the treatment groups can be considered comparable and results of the different treatments used in different groups can be compared. (There is no way at the time for the researchers to know which of the treatments is best. It is the patient's choice to be in a randomized trial or not.) (See also **Clinical Trials.**)

Regression (ree-gresh'un)—The state of growing smaller or disappearing; used to describe the shrinkage or disappearance of a cancer.

Remission (ree-mish'un)—The decrease or disappearance of evidence of a disease; also, the period during which this occurs.

Risk/Benefit Ratio—The relation between the risks and benefits of a given treatment or procedure. Institutional Review Boards (IRBs), located where the study is to take place, determine that the risks in a study are reasonable with respect to the potential benefits. It is also up to the patient to decide if it is reasonable for him or her to take part in a study.

Side Effect—A secondary and usually adverse effect, as from a drug or other treatment. For example, nausea is a side effect of some anticancer drugs.

Single Blind—(See **Double Blind.**)

Staging—Methods used to establish the extent of a patient's disease.

Standard Treatment—A treatment or other intervention currently being used and considered to be of proved effectiveness on the basis of past studies.

Study Arm—Patients in clinical trials are assigned to one part or segment of a study—a study "arm." One arm receives a different treatment from another.

Therapeutic (ther'a-pew'tik)—Pertaining to treatment.

Notes

Notes

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